HERCULES.
DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

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PRO135

PROJECT HERCULES: *A SYSTEMATIC REVIEW OF THE CONTENT AND STRUCTURAL VALIDITY OF PROS USED TO ASSESS QUALITY OF LIFE IN DUCHENNE MUSCULAR DYSTROPHY (DMD)*

Carlton J[1], Powell PA[1], Buckley Woods H[1], Mazzone P[1], Brazier JE[1]

[1] University of Sheffield, Sheffield, UK. scharr-outcomes@sheffield.ac.uk

	Content validity				Structural Validity
PROM	Relevance	Comprehensiveness	Comprehensibility	Quality of evidence	Rating of results
BDI				Very low	?
CALI				Low	?
DCGM-37				Low	?
EQ-5D-3L				Very low	?
FSS				Very low	?
GAD-7				Very low	?
HADS				Very low	?
HUI-2 / HUI-3 (15Q)				Very low	?
INQoL				Very low	?
KIDSCREEN-52				Low	?
KIDSCREEN-27				Low	?
KIDSCREEN-10				Low	?
LSIA				Moderate	?
MDCHILD				Low	?
PedsQL 3.0 DMD		?		Very low	?
PedsQL 3.0 MFS				Very low	?
PedsQL 3.0 NMM		?		Moderate	
PedsQL 4.0 GCS				Low	?
PedsQL 4.0 SF-15				Low	?
PHQ-9				Very low	?
PODCI				Very low	?
PSQI				Very low	?
SDQ				Very low	?
SF-36 v1.0				Very low	?
SWLS				Very low	?
WHOQOL-BREF				Very low	?

Figure 1. Satisfactory results; unsatisfactory results; inconsistent results; ? indeterminate

Objectives

Duchenne muscular dystrophy (DMD) is a rare genetic, progressive life-limiting paediatric neuromuscular disorder. Numerous **patient-reported outcome measures (PROs)** are administered to measure quality of life (QoL) in DMD, yet there has been no formal assessment of their validity. In this systematic review, we applied COSMIN criteria to evaluate the content and structural validity of PROs used to assess QoL in DMD.

Methods

Systematic searches were conducted across five academic databases (**EMBASE, MEDLINE, CINAHL, PsycINFO, and Cochrane Library**), supplemented by searches and citation tracking in Google Scholar. Full-text published articles containing evidence of content and/or structural validity of PROs assessing QoL in DMD, and/or articles on PRO development, were included. Evidence was synthesised and critically evaluated using established **COSMIN criteria**.

Results

60 eligible manuscripts featuring a PROM assessing QoL in DMD were identified. From these records, 40 PROs were extracted, and **26 PROs were evaluated using COSMIN**. Evidence on content and/or structural validity was extracted from 41 articles. Most PROs demonstrated low quality evidence and unsatisfactory or inconsistent validity. **The best performing PRO was the KIDSCREEN,** with an adequate rating for PRO design and a satisfactory content validity rating **(see Figure 1).**

Conclusions

Evidence is lacking on the content and structural validity of QoL PROs in DMD. We assessed the validity of 26 QoL PROs. Most PROs performed poorly against COSMIN criteria. In the absence of further work, **we recommend the use of the KIDSCREEN or the LSIA to assess QoL in young people with DMD**.

Acknowledgements

- Project HERCULES is an international multi-stakeholder collaboration led by Duchenne UK that is developing disease-level tools and evidence to support HTA and access decisions for new treatments for Duchenne Muscular Dystrophy.
- HERCULES is funded by Duchenne UK, Catabasis Pharmaceuticals Inc, Pfizer Inc, PTC Therapeutics, Roche, Sarepta Therapeutics Inc, Solid Biosciences, Santhera Pharmaceuticals Holding AG, Wave Lifesciences USA Inc.

Presented at ISPOR Europe 2019, Copenhagen, 2nd-6th November 2019